

CIRM-Funded Clinical Trial for Sickle Cell Gives Hope to People Battling the Disease

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Oakland, CA – Marissa Cors has lived with Sickle Cell Disease (SCD) for more than 40 years. The co-founder of The Sickle Cell Experience, an online platform designed to bring more awareness to Sickle Cell Disease, says it's hard, knowing that at any moment you may have to put your life on hold to cope with another attack of excruciating pain.

"It is incredibly frustrating to have a disease that is constantly disrupting and interfering with your life. The daily pain and fatigue make it difficult to have a normal life. You may be experiencing manageable pain one minute and then a crisis will hit - knocking you to the ground with horrible pain and requiring pain management and hospitalization. It makes going to school or having a job or even a normal adult relationship near impossible."

SCD is an inherited disease caused by a single gene mutation resulting in abnormal hemoglobin, which causes red blood cells to 'sickle' in shape. Sickling of red blood cells clogs blood vessels and leads to progressive organ damage, pain crises, reduced quality of life, and early death.

The disease affects around 100,000 Americans, mostly Black Americans but also members of the Latinx community. Marissa says coping with it is more than just a medical struggle. "Born into the cycle of fatigue, pain and fear. Depending on a healthcare system filled with institutionalized bias and racism. It is a life that is difficult on all facets."

CIRM is committed to trying find new treatments, and even a cure for SCD. That's why the CIRM Board recently awarded \$8,333,581 to Dr. David Williams at Boston Children's Hospital to conduct a gene therapy clinical trial for sickle cell disease. This is the second project that is part of an agreement between CIRM and the National Heart, Lung, and Blood Institute (NHLBI), part of the National Institutes of Health, to co-fund cell and gene therapy programs under the NHLBI's "Cure Sickle Cell" Initiative. The goal of this agreement is to markedly accelerate clinical development of cell and gene therapies to cure SCD.

"In recent years we have made impressive strides in developing new approaches to treating sickle cell disease," says Dr. Maria T. Millan, President & CEO of CIRM. "But we still have work to do. That's why this partnership, this research is so important. It reflects our commitment to pushing ahead as fast as we can to find a treatment, a cure, that will help all the people battling the disease here in the U.S. and the estimated 20 million worldwide."

The team will take a patient's own blood stem cells and insert a novel engineered gene to silence abnormal hemoglobin and induce normal fetal hemoglobin expression. The modified blood stem cells will then be reintroduced back into the patient. The goal of this therapy is to aid in the production of normal shaped red blood cells, thereby reducing the severity of the disease.

For Marissa, anything that helps make life easier will be welcome not just for people with SCD but their families and the whole community. "A stem cell cure will end generations of guilt, suffering, pain and early death. It will give SCD families relief from the financial, emotional and spiritual burden of caring someone living with SCD. It will give all of us an opportunity to have a normal life. Go to school, go to work, live with confidence."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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